

Overcoming the Legal and Regulatory Hurdles to Value-Based Payment Arrangements for Medical Products

December 2017

Overcoming the Legal and Regulatory Hurdles to Value-Based Payment Arrangements for Medical Products

White Paper

About the Duke-Margolis Center for Health Policy

The Robert J. Margolis, MD, Center for Health Policy at Duke University is directed by Mark McClellan, MD, PhD, and brings together expertise from the Washington, DC policy community, Duke University and Duke Health to address the most pressing issues in health policy.

The Center's mission is to improve health and the value of health care by developing and implementing evidence-based policy solutions locally, nationally, and globally. For more information, visit healthpolicy.duke.edu.

Authors

Gregory W. Daniel
Deputy Director, Duke-Robert J. Margolis, MD, Center for Health Policy and Clinical Professor, Fuqua School of Business, Duke University

Thomas Barker
Partner and Co-Chair, Healthcare Practice, Foley Hoag

Arti Rai
Elvin R. Latty Professor of Law and co-Director, Duke Law Center for Innovation Policy, Duke Law School, Duke University

Christian Springer
Associate, Foley Hoag LLP

Marianne Hamilton Lopez
Research Director, Duke-Robert J. Margolis, MD, Center for Health Policy, Duke University

Jane Kalinina
Policy Advisor, Foley Hoag LLP

Katie Greene
Research Associate, Duke-Robert J. Margolis, MD, Center for Health Policy, Duke University

Jingyuan Qian
Senior Research Assistant, Duke-Robert J. Margolis, MD, Center for Health Policy, Duke University

Mark B. McClellan
Director, Duke-Robert J. Margolis, MD, Center for Health Policy and Robert J. Margolis, MD Professor of Business, Medicine and Health Policy, Duke University

Advisory Group

Alan Balch
Chief Executive Officer, Patient Advocate
Foundation

Thomas Barker
Partner and Co-Chair, Healthcare Practice,
Foley Hoag

Amy Bassano
Acting Deputy Administrator for Innovation
and Quality & Acting Director of the Center
for Medicare and Medicaid Innovation,
Centers for Medicare and Medicaid Services

Jonathan Blum
Executive Vice President, Medical Affairs,
CareFirst BlueCross BlueShield

Marc Boutin
Chief Executive Officer, National Health
Council

Perry Bridger
Vice President, Global Value, Payer Access
and Public Policy, Edwards Lifesciences

Alexandra Clyde
Corporate Vice President of Global Health
Policy, Reimbursement and Health
Economics, Medtronic

Gavin Corcoran
Senior Vice President and Chief Medical
Officer, Allergan

Patrick Courneya
Executive Vice President and Chief Medical
Officer, Hospitals, Quality and Care Delivery
Excellence, Kaiser Permanente

Michael Goettler
Global President, Rare Disease Group, Pfizer
Innovative Health, Pfizer Inc.

Darin Gordon
President and CEO, Gordon & Associates,
LLC

Tim Hunt
Senior Vice President, Corporate Affairs,
Editas Medicine

Leigh Anne Leas
Vice President and US Country Head, Health
Policy, Novartis

Nick Leschly
Chief Executive Officer, bluebird bio

Jeff Marrazzo
Co-Founder and Chief Executive Officer,
Spark Therapeutics

Steve Miller
Senior Vice President & Chief Medical
Officer, Express Scripts

Joshua Ofman
Senior Vice President, Global Value, Access
& Policy, Amgen

Parashar Patel
Vice President, Global Health Policy, Boston
Scientific

Jonathan Perlin
President of Clinical Services and Chief
Medical Officer, Hospital Corporation of
America

Dana Gelb Safran
Chief Performance Measurement and
Improvement Officer/Senior Vice President,
Enterprise Analytics, Blue Cross Blue Shield
of Massachusetts

Craig Samitt
Executive Vice President and Chief Clinical
Officer, Anthem

Lewis Sandy
Executive Vice President, Clinical
Advancement, UnitedHealth Group

Michael Sherman
Chief Medical Officer and Senior Vice
President, Harvard Pilgrim Health Care

Surya Singh
Corporate Vice President and Chief Medical
Officer, Specialty Client Solutions,
CVSHealth

Susan Winckler
Chief Risk Management Officer, Leavitt
Partners/President, Leavitt Partners
Consulting

Linda Wiant
Chief, Medical Assistance Plan Division,
Georgia Department of Community Health

Contents

About the Duke-Margolis Center for Health Policy 1

Executive Summary 6

Introduction 9

Clarifying the Regulatory Landscape 12

FDA Regulation of Manufacturer Communications and Implications on Value-Based Payment Arrangements 12

Background on FDA’s Restrictions on Off-Label Promotion and Healthcare Economic Information 12

The 21st Century Cures Act and Recent Developments 14

FDA’s Guidance on HCEI Dissemination 15

Anti-kickback Statute 17

Background on the AKS 18

How the AKS Impedes VBP Arrangements 18

Identifying Existing Flexibility within the AKS for VBP Arrangements 20

Medicaid Best Price Requirement 23

Background on the MBP Requirement 23

How the MBP Requirement Impedes VBP Arrangements 24

Identifying Existing Flexibility within the MBP Requirement for VBP Arrangements 24

Regulatory and Legislative Solutions 27

Regulatory Proposals 27

FDA Regulation of Off-Label Communication 27

The Anti-Kickback Statute 29

Medicaid Best Price 31

Legislative Solutions 33

<i>FDA Regulation of Manufacturer Communications</i>	33
<i>Anti-Kickback Statute</i>	34
<i>Medicaid Best Price</i>	35
Conclusion	35

Executive Summary

Growing concerns over the high cost of new and innovative drugs and devices have led to increased interest in developing value-based payment (VBP) arrangements for these medical products, with the goal of achieving better outcomes at a lower overall cost than the current fee-for-service (FFS) system. These payment reforms aim to align pricing and/or payments more directly to evidence on outcomes and costs. As with VBP reforms for healthcare providers, implementation of VBP arrangements for medical products might be meaningfully viewed on a spectrum ranging from modest incremental FFS payment adjustments based on expected value, to contracts that involve substantial accountability and risk sharing for health outcomes and total costs for a population of patients. More advanced models rely on a combination of quality of care measures, clinical or patient-reported outcomes, utilization outcomes, and measures of spending intended to reflect value. Yet, while a growing share of health care payments are shifting to value-based approaches in other parts of health care, most VBP arrangements for drugs, devices, and other medical products are modest, involving limited performance-based adjustments in conjunction with predominantly FFS payments.

Some of the most notable hurdles to more advanced VBP arrangements for medical products involve current U.S. laws and regulations designed to address appropriate concerns about overuse, misuse, and excess spending associated with medical products in FFS payment systems. As we move away from FFS, these aspects of the current U.S. statutory and regulatory landscape not only complicate VBP implementation, but in some cases prevent their adoption.

Major concerns include:

- **Off-label communication restrictions:** VBP contracts require the bilateral exchange of a wide range of performance data among the manufacturers, payers, and providers involved in the contracts, often involving performance results and uses not yet on the product label, or products that are approaching the market but not yet with an approved label. Recent legislation and FDA regulatory action has addressed some of these issues, particularly involving economic outcomes. Yet, stakeholders have expressed concern about sharing such information for VBP contracts due to regulatory uncertainty about manufacturer communications and activities related to unapproved uses of approved or cleared medical products, impeding the widespread adoption of more sophisticated VBP arrangements.
- **Anti-kickback rules:** The Anti-Kickback Statute (AKS) is intended to prevent exchanges of value between manufacturers and other parties, which especially in FFS arrangements create risks of inappropriate care. While “safe harbors” have been developed for some activities, current rules do not consider payment models for medical products in which reimbursement depends mainly on measures of value and not on volume of sales. This environment is challenging for the implementation of VBP arrangements since the potential for increased value often depends on some degree of coordination and sharing of data, analytics, and other care improvement resources between the contracting parties.
- **“Best Price” and related pricing regulations:** A number of pricing rules for drugs, particularly the Medicaid Drug Rebate Program’s Best Price calculation (MBP) requirement, serve an important purpose in achieving low volume-based payments for drugs in Medicaid.

However, as currently formulated, failure to achieve desired outcomes in an advanced outcomes-based contract could trigger a higher rebate for all Medicaid utilization of the drug.

All of these barriers could benefit from clarification of current guidance, as well as from regulatory and legislative reforms to foster a more certain environment for the adoption of payments for medical products that shift substantially away from FFS. These shifts would align with major health care payment reform initiatives for providers, and could create more assurance and support for innovative products to deliver higher value to patients.

The Duke-Margolis Center for Health Policy, working with a broad-based Consortium—composed of patient advocates, payers, manufacturers, and providers, as well as experts on regulatory affairs, law, and policy — analyzed the legal barriers to meaningful VBP arrangements involving medical products, noting situations where stakeholders may be able to utilize the existing and emerging regulatory environment to develop and implement VBP arrangements.

Based on our analysis, we recommend that the Food and Drug Administration, Office of Inspector General, Centers for Medicare & Medicaid Services, and Congress take certain steps to advance legal certainty and incentivize the further development and adoption of meaningful VBP arrangements for medical products. Regulatory innovation to address these obstacles should match and support the innovations in 21st century technologies and healthcare organizations, by providing a clear pathway for aligning manufacturers and healthcare providers behind payment approaches that deliver better outcomes and avoid unnecessary healthcare costs.

Below is a summary of regulatory and legislative recommendations:

FDA Regulation of Manufacturer Communications

- Expand the scope and finalize the healthcare economic information (“HCEI”) draft guidance
- Implement a safe harbor for VBP arrangements
- Permit dissemination of HCEI related to investigational intended use
- Leverage 21st Century Cures authorities to facilitate development of VBP arrangements with RWE
- Promulgate regulations on off-label promotion
- Establish a safe harbor for pre-approval communication of HCEI
- Create regulatory certainty for off-label information to support value-based care models

Anti-Kickback Statute

- Reinterpret “volume or value of any referrals” in the context of VBP arrangements
- Reinterpret “Fair Market Value” in the context of VBP arrangements
- Revise existing safe harbors to facilitate VBP arrangements
- Establish a VBP arrangement safe harbor
- Establish clear policy direction with respect to VBP arrangements

Medicaid Best Price

- Reinterpret the bundled sales provision
- Clarify that rebates based on value negotiated by Medicaid managed care organizations do not trigger MBP
- Modify basis of measurement for MBP in the context of VBP arrangements through regulatory and/or legislative actions
- Establish Section 402 demonstrations for VBP arrangements
- Establish safe harbors for MBP
- Modify Center for Medicare and Medicaid Innovation (CMMI) statutory authority

Introduction

Biopharmaceutical and medical device innovation is rightly considered a critical pillar of the United States (U.S.) healthcare system. Rapid innovation in these industries promises to produce transformative therapies that will change the lives of patients with diseases previously considered intractable or in need of costly management of symptoms and complications. Simultaneously, however, payers and patients have grown increasingly cost-conscious. As a result, stakeholders are calling for new reimbursement models for biopharmaceutical and medical device products (together referred to as “medical products”) that help bring overall costs down and better align payments with the value they generate for patients.

The American healthcare system traditionally operates on a fee-for-service (FFS) payment basis, meaning that total payments are generally tied to the volume and intensity of services. This system can reward unnecessary and costly care, as revenue is tied to prioritizing volume and intensity of medical services rather than innovative care models that produce better outcomes, lower costs, and thus higher value. Further, FFS payments often lack support for coordination of care across a patient’s multiple providers and treatments, leading to inefficient and fragmented care that is not personalized to individual patient needs.

In contrast to FFS payment, “value-based payment (VBP)” arrangements for medical products are intended to align pricing and/or payments more closely to value across a population (i.e., outcomes relative to costs). As with payment reforms for healthcare providers, implementation of VBP arrangements for medical products might be meaningfully viewed on a spectrum, beginning with payments that remain FFS-based but that are adjusted based on evidence of expected value, such as with indication-based pricing. This spectrum extends to outcomes-based contracts (OBCs) that link payments to that product’s actual performance or value in a patient or a population. OBCs can potentially allow stakeholders, including payers, manufacturers, providers, and health systems, to align their financial interests directly with the performance of the medical product. These payments can encourage greater shared efforts to improve outcomes for the patient population treated, including such steps as developing better evidence on how treatments work and can be improved in real-world settings, and advancing innovative care models based on better alignment of manufacturers and providers around health outcomes.

With OBCs, accountability for results is based on clinical or patient-reported outcomes, utilization outcomes, measures of spending, and/or quality of care measures.¹ These measures would inform a results- or value-based adjustment to payments. Although the terminology associated with these types of agreements can vary (i.e. “risk-sharing agreements,” “outcomes-based agreements,” “performance-based agreements,” “accountable care payments,” etc.),

¹ Garrison, L. P. J., Carlson, J. J., Bajaj, P. S., Towse, A., Neumann, P. J., Sullivan, S. D., Westrich, K., Dubois, R. W. (January 01, 2015). Private sector risk-sharing agreements in the United States: trends, barriers, and prospects. *The American Journal of Managed Care*, 21, 9, 632-40.

they all share a common feature of linking payment for therapies or interventions to measures of actual results or other outcomes achieved in a patient population. These models can vary in terms of how much and how comprehensively they shift from volume- to value-based payment. For example, more straightforward OBCs might include manufacturer “warranties” that involve full upfront payment with some rebates for the product tied to easier-to-collect measures such as medication adherence or hospitalization rates in the population receiving the product. More advanced OBCs might involve linking most or all payments to a more comprehensive set of outcome and spending measures, including clinical measures and/or patient-reported outcomes, as well as impact on total cost of care. The movement of OBCs along the continuum from simple to advanced represents an increasing shift away from FFS.

While medical product payment contracts have been typically negotiated between payers and medical product manufacturers, the growing prominence of VBP models has led to increasing interest in aligned VBP arrangements involving manufacturers and providers. These latter VBP arrangements potentially allow involved parties to align their financial interests and share accountability in overall patient outcomes. VBP arrangements that align across providers, payers, and manufacturers could prove especially effective if adopted by health systems or different providers that are virtually aligned through their own VBPs, giving them the ability to coordinate patient care. As experience and capacities to implement such VBP arrangements evolves, contracts could increasingly reflect close alignment between provider and medical product payments with the value produced for patients and the health system. Many stakeholders view VBP arrangements as potentially driving more efficient healthcare delivery, with reductions in overall costs while improving patient outcomes.

VBP arrangements are still in their infancy. Their slow development and adoption in the U.S. is attributable to numerous factors, including the lack of consensus on what constitutes “value,”² agreement on best measures, availability and sharing of data, and other operational challenges. Some of the most notable hurdles involve current U.S. laws and regulations designed to address appropriate concerns about overuse, misuse, and excess spending associated with medical products in FFS payment systems. These aspects of the current U.S. statutory and regulatory landscape not only complicate VBP implementation, but in some cases prevent their adoption. In particular, stakeholders have frequently identified at least three types of regulatory and

² Defining “value” continues to be a very hotly debated topic and the discussion is far from consensus. Some organizations have put forward methodologies for defining and measuring value. Most notable among them is the Institute for Clinical and Economic Review (ICER), which was referenced by the Center for Medicare & Medicaid Services in its now-defunct Part B Drug Payment Model Proposal. See “Medicare Program; Part B Drug Payment Model,” 81 Fed. Reg. 13229, 13243 (March 11, 2016). Ideally, these types of analyses would inform “value-based payment (VBP) arrangements”, but they need not to.

statutory `barriers^{3,4,5} as posing significant limitations on the expanded adoption of VBP arrangements. These are (1) the U.S. Food and Drug Administration’s (FDA) regulation of manufacturer promotional communications^{6,7,8,9,10} (2) the Anti-Kickback Statute (AKS),¹¹ and (3) the Medicaid Drug Rebate Program’s Best Price calculation (MBP) requirement.^{12,13,14,15,16,17}

The purpose of this White Paper is to describe these three legal barriers to meaningful VBP arrangements involving medical products, and to identify specific, practical steps to address them.¹⁸ These hurdles are increasingly restrictive as payment models move further from FFS and toward arrangements where revenues depend not on volume of sales but on measures of value. Consequently, existing statutes and regulations will need to be clarified or modified to enable meaningful shifts from FFS to value-based reimbursement for medical products. In terms of price-reporting requirements, while MBP is the primary focus of our discussion, we

³ 42 U.S.C. § 1396r-8(c)(1)(C).

⁴ Ward, A. S., Linthicum, M., Drozd, M., Silverstein, A. R., & Vandigo, J. (2016, November 4). Regulatory, Legal Uncertainties Are Barriers To Value-Based Agreements For Drugs. *Health Affairs Blog*. Retrieved April 11, 2017, from <http://healthaffairs.org/blog/2016/11/04/regulatory-legal-uncertainties-are-barriers-to-value-based-agreements-for-drugs/>

⁵ *Barriers to Value-Based Contracts for Innovative Medicines*. PhRMA Member Survey Results. March 2017.

⁶ Food and Drug Administration Modernization Act of 1997 § 102, 42 U.S.C. § 4332 (1997).

⁷ FDA Federal Register Notice: Manufacturer communications Regarding Unapproved Uses of Approved or Cleared Medical Products, Federal Register. 82(12):6367 (January 19, 2017).

⁸ FDA Guidance: United States Government. U.S. Food and Drug Administration. (2017). *Medical Product Communications that are Consistent with the FDA-Required Labeling*. Silver Spring, Maryland. FDA.

⁹ FDA Memorandum: United States Government. U.S. Food and Drug Administration. (2017). *Public Health Interests and First Amendment Considerations Relation to Manufacturer Communication Regarding Unapproved Uses of Approved or Cleared Medical Products*. Silver Spring, Maryland. FDA.

¹⁰ Recommendations included in NEHI, PhRMA, and *Health Affairs* publications.

¹¹ 42 U.S.C. § 1320a-7b.

¹² 42 U.S.C. § 1396r-8.

¹³ Kelly, C. (November 6, 2016). US Outcomes-Based Contracts: Big Uptick In Interest, But Not Execution. Available at: <https://invivo.pharmamedtechbi.com/IV004953/US-OutcomesBased-Contracts-Big-Uptick-In-Interest-But-Not-Execution>

¹⁴ *Rewarding Results: Moving Forward on Value-Based Contracting for Biopharmaceuticals*. The Network for Excellence in Health Innovation. March 2017

¹⁵ Eli Lilly and Company and Anthem. (January 29, 2016). Promoting Value-Based Contracting Arrangements. Available at: <https://lillypad.lilly.com/WP/wp-content/uploads/LillyAnthemWP2.pdf>

¹⁶ Margulies R. CMS releases guidance to states and manufacturers on Medicaid value based purchasing arrangements. Foley Hoag LLP. July 15th, 2016. Accessed at: [<http://www.medicaidandthelaw.com/2016/07/15/cms-releases-guidance-to-states-and-manufacturers-on-medicaid-value-based-purchasing-arrangements/>]

¹⁷ “States and the rising cost of Pharmaceuticals: A Call to Action.” National Academy for State Health Policy, October 2016.

¹⁸ This paper focuses primarily on VBP arrangements that take the form of OBCs. We acknowledge that there are important advances occurring in the use of indication-specific payment models that seek to align payment with a medical product’s indication-specific efficacy, as established through existing clinical trial data. Although these types of payment models also reflect a move away from traditional FFS and share similar legal and regulatory hurdles to their design and implementation as OBCs, they are beyond the scope of this paper.

also briefly address similar issues with average sales price (ASP) and average manufacturer price (AMP).

This White Paper also notes situations where stakeholders may be able to utilize the existing and emerging regulatory environment to develop and implement VBP arrangements. Even in these situations, concerns may remain about the compliance risk associated with pursuing such arrangements without further clarifications or modifications in the flexibility of the current statutory and regulatory environment to support VBP arrangements. Therefore, we conclude with proactive regulatory and legislative recommendations to foster a more certain and supportive environment for a substantial shift from FFS to VBPs for medical products.

Clarifying the Regulatory Landscape

The discussion that follows aims to identify how the three key regulatory concerns operate to impede VBP arrangements. The discussion demonstrates that although there is limited flexibility within the existing regulatory regime that can be utilized to develop and adopt VBP arrangements, regulatory uncertainty and compliance risk will continue to be an important limiting factor for manufacturers and other parties interested in pursuing VBP arrangements.

FDA Regulation of Manufacturer Communications and Implications on Value-Based Payment Arrangements

A fundamental principle underlying the success of any VBP arrangement is the ability of the contracting parties to communicate, to share reliable, if imperfect, evidence. Such evidence is used to assess “value” and informs negotiations that set parameters, which might include data on costs and comparative effectiveness, product utilization impact reports, clinical outcomes, and other quality and economic metrics. This type of information includes real-world evidence (RWE) developed following a product’s approval, and may be derived from clinical practice and other sources of real-world data (RWD).

Due to the importance of this bilateral exchange of data, FDA’s regulatory authority over medical product labeling and regulation of manufacturer promotional communications has important implications for the successful development and execution of VBP arrangements. For this reason, stakeholders have expressed concern that regulatory uncertainty surrounding the FDA’s rules and policies on manufacturer communications and activities related to unapproved uses of approved or cleared medical products (commonly known as “off label” uses) impedes the widespread adoption of VBP arrangements.

Background on FDA’s Restrictions on Off-Label Promotion and Healthcare Economic Information

Under FDA’s regulation of manufacturer communications, a manufacturer’s ability to relay information not contained in the FDA-approved labeling is restricted and subject to an array of rules and guidance documents. Information found in the FDA-approved labeling is generally derived from adequate and well-controlled randomized clinical trials and other “substantial

evidence” reviewed at the time of product approval. This information is often limited to clinical measures, such as lab values or other markers of clinical benefit, which may be less meaningful for purposes of structuring VBP arrangements,¹⁹ and contain less information on economic outcomes, health outcomes in subpopulations relevant to a particular payer, or comparisons to other treatment approaches in practice. Historically, there has been uncertainty about whether sharing data on real-world outcomes that are meaningful for defining value but are absent in the labeling, such as reduced hospitalization rates, could be perceived as promoting an unapproved use of a product. Such communications, if viewed as promotional in nature, could subject the manufacturer to FDA enforcement action and other liability risk.²⁰

Recognizing the importance of this information to the payer community, in 1997 Congress created a safe-harbor to permit manufacturers to proactively communicate “healthcare economic information” (“HCEI”) to “a formulary committee, or other similar entity,” provided the HCEI is based on “competent and reliable scientific evidence” (“CARSE”) and “directly relates” to an approved indication.²¹ While Congress intended to allow manufacturers to share cost-effectiveness information and other “economic consequence” information,²² confusion among stakeholders about how to utilize the safe harbor and a lack of clear guidance from FDA on what type of data met the definition of HCEI and the appropriate scope of the audience to receive HCEI has hampered utilization of the safe-harbor for nearly twenty years following its creation.²³

In addition to confusion among stakeholders over what type of information qualifies as HCEI and how the safe harbor should be utilized, a series of First Amendment court decisions cast additional uncertainty on the legal validity of FDA’s rules and policies governing manufacturer off label communications. Following these cases in which the courts ruled against FDA,²⁴ some stakeholders believe FDA’s authority to regulate off-label communication that is truthful and non-misleading is limited by the First Amendment.

¹⁹ See e.g., Ward, A. S., Linthicum, M., Drozd, M., Silverstein, A. R., & Vandigo, J. (2016, November 4). Regulatory, Legal Uncertainties Are Barriers To Value-Based Agreements For Drugs. *Health Affairs Blog*. Available at <http://healthaffairs.org/blog/2016/11/04/regulatory-legal-uncertainties-are-barriers-to-value-based-agreements-for-drugs/>. Note that FDA labeling is not always limited to intermediate outcomes and could contain other information such as efficacy endpoints and outcomes that could be clinically relevant and “real-world” applicable.

²⁰ For example, stakeholders have also voiced concerns that off-label communications issue may also implicate False Claims Act liability.

²¹ Section 114 of the Food and Drug Administration Modernization Act (FDAMA) of 1997.

²² Under Section 114 of FDAMA, HCEI was defined as “any analysis that identifies, measures, or compares the economic consequences, including the costs of the represented health outcomes, of the use of a drug to the use of another drug, to another health care intervention, or to no intervention.”

²³ See e.g., AMCP Partnership Forum: FDAMA Section 114—Improving the Exchange of Health Care Economic Data.

²⁴ See *United States v. Caronia*, 703 F.3d 149 (2d Cir. 2012) (Court explicitly rejected that accurate speech about off-label information could be basis for criminal prosecution); *Amarin Pharma, Inc. v. FDA*, 119 F. Supp. 3d 196 (S.D.N.Y. 2015) (Milestone settlement between FDA and Amarin permitting off-label promotion of an unapproved use); *United States v. Vascular Solutions, Inc.*, No. 14-0926, ECF No. 286 (verdict form) (W.D. Tex. Feb. 26, 2016) (Acquittal of all criminal charges brought against a medical device manufacturer and CEO based on the alleged off-label promotion of a medical device).

In this context, FDA held a two-day public meeting in November of 2016 and requested feedback as part of a reexamination of its promotional rules and policies relating to firm communications regarding off-label uses.²⁵ Public comment and testimony from many payers and public health decision makers advocated for policies that permit communication of broader and non-misleading information about a product pre-approval to facilitate budget planning and to support further adoption of VBP arrangements.²⁶

The 21st Century Cures Act and Recent Developments

As FDA continues to review its rules and policies related to off-label communications, recent developments have clarified how manufacturers may communicate HCEI information about an approved drug to payers, which may support communications related to VBP arrangements.

In December 2016, Congress passed the 21st Century Cures Act (“Cures Act”),²⁷ which modified the Food and Drug Administration Modernization Act (“FDAMA”) Section 114 definition of HCEI to include certain clinical outcomes data and analyses; expanded the scope of audience with whom HCEI may be shared; and changed the requirement that HCEI had to “directly relate” to an approved indication to a new standard that HCEI must “relate to” an approved indication. The law maintained the standard that qualifying HCEI must be based on CARSE. Manufacturers may now rely on this safe harbor to proactively share information with payers, including in VBP arrangement negotiations, that was previously believed to be outside the scope of FDAMA’s definition of HCEI, such as clinical data and assumptions underlying the HCEI analysis.

Following the passage of the Cures Act, there have been other important developments in this regulatory area. For instance, in January 2017, FDA released two draft guidance documents²⁸ and a memorandum on First Amendment considerations to “clarify the agency’s thinking” on manufacturer communications that are “consistent with” the product’s label; to address HCEI dissemination after passage of the Cures Act; and to solicit further public comment on FDA’s rules and policies on manufacturer off-label communications. Stakeholders are waiting for FDA to finalize its review of its policy and guidance documents, as the scope and content of the final HCEI guidance document will influence the underlying information that can be used as a basis for the VBP contracts.

²⁵ 81 Fed. Reg. 60299 (Sept. 1, 2016).

²⁶ See generally U.S. Food and Drug Administration, Manufacturer Communications Regarding Unapproved Uses of Approved or Cleared Medical Products Part 15 Public Hearing, November 9-10, 2016, Transcripts, available at <https://www.fda.gov/downloads/NewsEvents/MeetingsConferencesWorkshops/UCM532489.pdf> and <https://www.fda.gov/downloads/NewsEvents/MeetingsConferencesWorkshops/UCM532491.pdf>; see also Manufacturer Communications Regarding Unapproved Uses of Approved or Cleared Medical Products, Docket ID: FDA-2016-N-1149, available at <https://www.regulations.gov/docket?D=FDA-2016-N-1149>.

²⁷ Pub. L. 114-255.

²⁸ See Draft Guidance on Manufacturer Communications That Are Consistent With The FDA-Required Labeling – Q&A (January 2017); Draft Guidance on Drug and Device Manufacturer Communications With Payors, Formulary Committees, and Similar Entities – Q&A (January 2017)(hereinafter “draft payor guidance”).

FDA's Guidance on HCEI Dissemination

In the draft payer guidance, FDA defines the scope of data meeting the HCEI definition under the Cures Act²⁹ and states “HCEI pertains to the economic consequences related to the *clinical outcomes* of treating a disease (or specific aspect of a disease) or of preventing or diagnosing a disease[,]” which includes both the cost-benefit analysis to “alternative options (including the use of another drug) or to no intervention.”³⁰ FDA notes that this information “can be presented in a variety of ways that can include, but are not limited to, an evidence dossier, a reprint of a publication from a peer-reviewed journal, a software package comprising a model with user manual, or a budget-impact model.”³¹ This includes information such as differences in dosing schedules of an approved dosage form, burden of illness factors, practice-setting changes, and duration of treatment. Under the draft guidance, all are provided as examples of HCEI “related to” the approved indication. An example provided by FDA that would not be considered sufficiently related to the approved indication is an “[a]n economic analysis of *disease course modification* related to use of a drug that is approved only to treat the *symptoms* of the disease.”³²

It is notable that the draft guidance fails to address communication of HCEI regarding currently unapproved uses of approved drugs, limiting discussion to approved indications only. This has implications for various VBP designs, as economic research about disease costs and outcomes often goes beyond the scope of approved indications.³³ For example, consider the use of RWE, such as observational retrospective studies or claims data that provides information about outcomes involving a drug prescribed “off-label” for a new population. Under FDA’s draft guidance, it is unclear if this type of communication would fall outside the scope of permissible HCEI.³⁴ If finalized without changes, this ambiguity would have implications for VBP

²⁹ HCEI means “any analysis (including the clinical data, inputs, clinical or other assumptions, methods, results, and other components underlying or comprising the analysis) that identifies, measures, or describes the economic consequences, which may be based on the separate or aggregated clinical consequences of the represented health outcomes, of the use of a drug. Such analysis may be comparative to the use of another drug, to another health care intervention, or to no intervention. . . . Such term does not include any analysis that relates only to an indication that is not approved under section 505 or under section 351(a) of the Public Health Service Act for such drug.” See Section 502(a) of the Federal Food, Drug, and Cosmetic Act. (emphasis added). Note that while the definition of HCEI explicitly applies to drugs, many stakeholders argue that the same principals and policies apply with respect to medical devices and HCEI communications. For example, the draft guidance includes examples and references that are specifically relevant to the medical device industry. As a result, stakeholder comments to the draft payor guidance “encourage FDA to explicitly discuss the related scope of the Guidance specifically for medical devices.” See *e.g.*, The Advanced Medical Technology Association, Comment Submission, Docket No. FDA-2016-N-1307, available at

[file:///wasfhprof01/home/ykalinina/Downloads/Comment from Advanced Medical Technology Association Adv aMed%20\(8\).pdf](file:///wasfhprof01/home/ykalinina/Downloads/Comment%20from%20Advanced%20Medical%20Technology%20Association%20Adv%20aMed%20(8).pdf)

³⁰ HCEI draft guidance at 3 (emphasis added).

³¹ *Id.* at 4.

³² Draft payor guidance at 8. (emphasis added).

³³ See Section 502(a)(“ . . . Such term does not include any analysis that relates only to an indication that is not approved under section 505 or under section 351(a) of the Public Health Service Act for such drug.”).

³⁴ In the draft payer guidance FDA notes that “HCEI analyses derived from studies in patient populations that are not within the indicated patient population are not related to the approved indication of the drug. For example, an

arrangements such as those that wish to measure performance on the actual use of the product, which could raise difficult risk decisions for establishing baseline payments and other metrics. Further, structuring indication-based pricing for products that have numerous off-label uses would likely face challenges under FDA's draft guidance that could discourage their implementation.³⁵

FDA further clarifies the "appropriate scope of the audience" for HCEI communications and states that payers, formulary committees, drug information centers, technology assessment panels, pharmacy benefits managers, and "other multi-disciplinary entities that review scientific and technology assessments to make drug selection, formulary management, and/or coverage and reimbursement decisions on a population basis for health care organizations" may receive HCEI.³⁶ However, the draft guidance states that the guidance would not apply to dissemination of HCEI to other audiences, such as "health care providers who are making individual patient prescribing decisions."³⁷ While it is generally understood that healthcare providers should not be precluded from receiving HCEI as long as the Cures statutory criteria is met and the provider is receiving the information as part of their role in an appropriate entity receiving the HCEI³⁸ the draft guidance does not explicitly discuss sharing HCEI with healthcare providers or other parties engaged in VBP contracting activities that involve a manufacturer's product. Instead, the draft guidance expressly states that FDA "does not regulate the terms of contracts between firms and payors,"³⁹ even though such limits on HCEI exchange could hinder emerging VBP arrangements.

With respect to implementation of this guidance for HCEI dissemination, FDA notes that if a manufacturer disseminates HCEI to only appropriate audiences, and the HCEI is related to an FDA approved indication and one that is based on CARSE, FDA will not consider this communication as false or misleading off-label promotion or evidence of promoting a new intended use for the product. FDA notes that it will find that an indication is based on CARSE

analysis regarding the treatment of cystic fibrosis (CF) in patients with any mutation in the CF gene would not be considered to relate to the approved indication for a drug approved to treat only one specific CF gene mutation." See payer draft guidance at 8.

³⁵ See e.g., Institute for Clinical and Economic Review (ICER), *Indication-Specific Pricing of Pharmaceuticals in the United States Health Care System*, A Report from the 2015 ICER Membership Policy Summit (March 2016)(hereinafter Indication Pricing Report)("Manufacturers can only negotiate reimbursement contracts for FDA approved indications. Drugs that have significant off-label uses, including ones that may be supported by research, guidelines and compendia, are unlikely to be suitable candidates for indication-specific pricing since a decision must be made regarding which price will be used for off-label uses, and manufacturers cannot enter into contract negotiations that in any way give the perception of promoting off-label use. Indication-specific pricing discussions should therefore focus on drugs that have a low risk for off-label use beyond existing indications."). Note the Indication Pricing Report offers potential solutions to this obstacle, noting "payers and manufacturers can address these concerns by selecting drugs for ISP that have minimal off-label use, by applying indication-specific price adjustments only to labeled indications, and by using a weighted average approach to ISP." *Id* at 17.

³⁶ See Draft payer guidance at 4.

³⁷ *Id.*

³⁸ *Id.*

³⁹ *Id.* at 15.

only “if the HCEI has been developed using generally-accepted scientific standards, appropriate for the information being conveyed, that yield accurate and reliable results.”⁴⁰ Further, If HCEI includes material differences from the FDA-approved labeling, it must present “a conspicuous and prominent statement describing any material differences between the health care economic information and the labeling approved for the drug.”⁴¹

Finally, to respond to stakeholders’ concerns regarding pre-approval communications and needs for budget and formulary management, FDA’s draft guidance also permits communicating “unbiased, factual, accurate, and non-misleading” data about an investigational drug and device product, subject to conditions laid out in the draft guidance.⁴² This safe harbor would apply to communications involving the drug class or device design, information on the indication sought, information from clinical studies about endpoints, factual clinical trial results, anticipated timeline for FDA approval, product pricing information, marketing strategies, and patient support programs.⁴³ However, many stakeholders have raised questions about the extent and type of information this might entail and have requested additional clarity from FDA.⁴⁴

The draft guidance is a widely welcomed clarification of what constitutes appropriate proactive communication of HCEI with payers that helps mitigate some of the historic challenges associated with navigating FDA’s rules and policies concerning manufacturer communications with payers. However, the draft guidance fails to address pre-approval discussions of unapproved indications of a marketed product, leaves certain ambiguity about the types of other communications that may be appropriate for VBP arrangement negotiations as part of setting contractual terms, and leaves open questions about the extent and substance of information that may be shared about an investigational product prior to its approval. Therefore, if the draft guidance is adopted without revisions, it would fail to address significant current regulatory uncertainty around VBP arrangements.

Anti-kickback Statute

Federal fraud and abuse law consists of several distinct but interrelated sources of law, including the False Claims Act, the Physician Self-Referral Law, the Exclusion Statute, the Civil Monetary Penalties Law, and the Anti-Kickback Statute (AKS). Depending on the type of VBP arrangement involved, some of these laws may be more relevant than others. However, compliance with the AKS raises substantial concerns for VBP contracts given the law’s broad reach.⁴⁵

⁴⁰ *Id.* at 9.

⁴¹ *Id.* at 5.

⁴² See generally HCEI draft guidance at 15-18.

⁴³ *Id.*

⁴⁴ See U.S. Food and Drug Administration, Public Docket ID: FDA-2016-D-1307.

⁴⁵ This is particularly true post-ACA. The ACA introduced language at 42 U.S.C. § 1320a-7b(g) which provides that an AKS violation automatically establishes a False Claims Act violation as well.

Background on the AKS

The AKS prohibits anyone from knowingly and willfully offering, making, soliciting, or receiving any payment in return for (1) referring an individual to another person or entity for the furnishing of any item or service reimbursed by Medicare, Medicaid or some other federal healthcare program, or (2) recommending or arranging for the ordering of any service reimbursed by Medicare, Medicaid, or some other federal healthcare program.⁴⁶ A violation of the AKS is punishable up to five years imprisonment and/or a \$25,000 fine.⁴⁷ Further, a violation of the AKS excludes the involved parties from participation in all federal healthcare programs.⁴⁸

While recognizing its importance in preventing improper incentives for prescribing and treatment, Congress has acknowledged the need to limit the breadth of the AKS numerous times. In 1987, Congress directed the Secretary of Health and Human Services (HHS) to create safe harbors.⁴⁹ The Office of Inspector General (OIG) has established numerous safe harbors over the years, but they are typically drafted narrowly and contain highly technical requirements. In 1996, Congress acknowledged that the safe harbors may not offer sufficient legal certainty to stakeholders and it further authorized the OIG to issue “Advisory Opinions” (AOs) to parties concerned that a given arrangement or conduct would violate the anti-kickback prohibitions.⁵⁰ Advisory opinions are binding only on HHS and the party requesting the opinion.

How the AKS Impedes VBP Arrangements

Because the AKS was promulgated prior to the rise of VBP arrangements, its safe harbors do not generally contemplate arrangements that shift away from volume-based payment to payment models in which reimbursement depends on measures of value. This is challenging for the implementation of VBP arrangements since their potential for increased value often depends on some degree of coordination and sharing of resources between the contracting parties. Reflecting this, the Center for Medicare & Medicaid Services (CMS) and the OIG jointly issued broad fraud and abuse waivers for the viable operation of the Medicare Share Savings Program (MSSP) and its principal cost-saving mechanism known as an Accountable Care Organization (ACO).⁵¹

Outside the context of providers in certain alternative payment models, however, stakeholders that desire to enter into VBP arrangements remain subject to the AKS’ prohibitions. Many argue that other existing safe harbors offer inadequate assurances that VBP arrangements will not be penalized under the AKS. For illustrative purposes, this White Paper focuses on highlighting the limitations of the warranty safe harbor, the electronic health records (EHR) safe harbor, and the

⁴⁶ 42 U.S.C. § 1320a-7b.

⁴⁷ 42 U.S.C. § 1320a-7b(1)(B).

⁴⁸ 42 U.S.C. § 1320a-7.

⁴⁹ 42 U.S.C. § 1320a-7B(b)(3)(E).

⁵⁰ 42 U.S.C. § 1320a-7d(b).

⁵¹ “Medicare Program; Final Waivers in Connection With the Shared Savings Program,” 80 Fed. Reg. 66726 (Oct. 29, 2015).

discount safe harbor. Although these safe harbors limit VBP arrangements in specific ways, we emphasize that the nature of these limitations (i.e. limitations designed to discourage coordination and collaboration) generally apply to all of the other safe harbors as well.

To illustrate the inadequacy of the warranty safe harbor, consider a VBP arrangement between a device manufacturer and a provider wherein the manufacturer would agree to reimburse the provider's costs associated with hospitalization (or other medical services) resulting from a defective device or a device that fails to produce agreed-upon outcomes. This arrangement implicates the AKS because OIG considers the reimbursement of potential ancillary costs to be "remuneration" that can influence providers to purchase the device. Importantly, it would not meet the technical requirements of the warranty safe harbor because the safe harbor protection does not extend to costs beyond those associated with the replacement of the defective device itself, much less to ancillary costs of a product's failure to meet agreed-upon health outcomes.⁵² This limitation is critical because VBP arrangements for medical devices and drugs often include assurances that the manufacturer will share in accountability for any ancillary additional costs associated with unintended consequences or sub-par performance. To fit under the warranty safe harbor, however, a guarantee would need to be limited to the cost of the device itself and not include any ancillary costs otherwise borne by the provider or a payer. The guarantee could also not be offered in the event of sub-optimal performance. The effect that these limitations have on VBP arrangements should be clear: medical product manufacturers seeking to guarantee enhanced value to providers by bearing financial risk exposes both parties to AKS liability.

Another example is in the context of the EHR safe harbor. Consider a VBP arrangement between a drug manufacturer and provider wherein the drug manufacturer would provide EHR software and related analytic support to assist in care management or tracking of relevant clinical outcome data relating to the manufacturer's drug. Such technology may be vital to the proper functioning of a VBP arrangement, since it resolves operational challenges related to tracking and analyzing data. Yet, it could also potentially violate the AKS because OIG would consider the software and analytic support to be "remuneration" that could induce the provider to purchase the manufacturer's drug.⁵³ This VBP arrangement would likely not satisfy the EHR safe harbor's requirement that the receipt of the technology not be conditioned on doing business with the manufacturer.⁵⁴ Moreover, this type of VBP arrangement could involve restrictions on the EHR system's interoperability, which may violate the EHR safe harbor's requirement of interoperability.⁵⁵ Together, these safe harbor limitations restrict the creation

⁵² 42 C.F.R. § 1001.952(g)(4) ("[R]emuneration to any individual or entity...[is limited] to the cost of the item itself.")

⁵³ In AO 12-19, the OIG found a specific proposed arrangement, wherein software was provided at more than nominal cost and was not interoperable, would generate prohibited remuneration under the AKS and that the OIG could impose administrative sanctions. See OIG Advisory Opinion 12-19 (Dec. 2012), available at <https://oig.hhs.gov/fraud/docs/advisoryopinions/2012/AdvOpn12-19.pdf>.

⁵⁴ 42 C.F.R. § 1001.952(y)(4).

⁵⁵ 42 C.F.R. § 1001.952(y)(3).

of VBP arrangements that operationally facilitate data collection and analysis relating to a particular therapy or technology.

Finally, certain aspects of the discount safe harbor also limit the adoption of VBP arrangements. At the outset, discounts may come in many forms. For example, consider a VBP arrangement between a drug manufacturer and a hospital wherein the manufacturer agrees to offer a discount that would depend on the satisfaction of specified health outcomes within a five-year timeframe (i.e. relapse of the disease in year three would lead to a 30 percent discount on the full price of the product). This type of VBP arrangement could implicate the AKS because OIG could consider the promise of a discount based on the occurrence of certain outcomes to be “remuneration” that could induce a provider’s purchase of the drug. Moreover, this VBP arrangement would not be protected by the discount safe harbor because the safe harbor requires that the hospital claim the benefit of the discount within a maximum of two years, not a maximum of five years.⁵⁶ This time horizon limitation may be more pronounced in the case of VBP arrangements involving gene therapies and other potentially curative therapies, since the VBP arrangement would likely involve an extended time horizon to account for the possibility that the genetic disease may reemerge at a much later point in a patient’s life. Moreover, it is unclear whether the discount safe harbor extends to arrangements between manufacturers and payers, given the “buyer” categories set forth in the regulations.⁵⁷

These specific examples illustrate how the AKS generally operates to limit VBP arrangements. This inhibiting interaction between the AKS and VBP arrangements reflects the AKS’ understandable focus on mitigating perverse incentives to prioritize volume over value that may emerge in a FFS payment system. For VBP arrangements that represent a substantial shift away from payment arrangements that reward high volume and total spending, the fraud and abuse framework of the AKS needs to accommodate the potential of VBP arrangements to help achieve the AKS goals of avoiding unnecessary program costs and improving outcomes.

Identifying Existing Flexibility within the AKS for VBP Arrangements

Although the current AKS framework may impede the development and expanded adoption of VBP arrangements, some flexibility may exist to enable such arrangements to proceed under certain circumstances. This potential flexibility includes the OIG’s “prudential” approach to evaluating contractual arrangements that may violate the AKS. In addition, some VBP arrangements are not implicated by the AKS, because it applies only to “federal health care programs.”

Parties implicate the AKS when the goods or services involved in the “remuneration” are paid “in whole or in part under a Federal health care program....”⁵⁸ Although “federal health care program” is defined broadly by the statute,⁵⁹ the statutory definition does not extend to the commercial sector. Consequently, a VBP arrangement that is limited to *private* providers,

⁵⁶ 42 C.F.R. § 1001.952(h)(1)(ii)(B).

⁵⁷ 42 C.F.R. § 1001.952(h)(1)-(3).

⁵⁸ 42 U.S.C. § 1320a-7b(b).

⁵⁹ 42 U.S.C. § 1320a-7b(f).

manufacturers, and payers would generally not implicate the AKS.⁶⁰ Stakeholders should recognize that the AKS does not generally impede the development and adoption of VBP arrangements as long as the contracting parties structure their VBP arrangement to exclude items and services reimbursable under a federal healthcare program.^{61,62}

Furthermore, it is an open question whether Qualified Health Plans (QHPs) that are eligible for Federal subsidies in state insurance exchanges are subject to the AKS. HHS' latest interpretation of "federal health care programs" does not include QHPs purchased through the Health Insurance Marketplaces (Marketplaces). In a letter dated October 30th, 2013 to Rep. Jim McDermott, then Secretary Kathleen Sebelius asserted that "The Department of Health and Human Services does not consider QHPs, other programs related to the Federally-facilitated Marketplace, and other programs under Title I of the Affordable Care Act to be federal healthcare programs."⁶³ While such a letter may not offer adequate assurances to some, others may view this interpretation as suggesting that the Marketplaces may contain at least some flexibility to enter into innovative VBP arrangements without generally implicating the AKS.

Even within the confines of the AKS, stakeholders should not overlook the flexibility exercised by the Office of the Inspector General (OIG) in its enforcement discretion, as manifested in its Advisory Opinions. There are some arrangements that the OIG simply does not consider to pose a sufficient fraud and abuse risk as to warrant enforcement of the AKS, even when those arrangements clearly implicate the AKS *and* fall outside a safe harbor. For example, OIG determined that a proposed arrangement fell outside the protection of the warranty safe harbor because it involved the "bundling" of products and services, yet the OIG allowed the arrangement because the warranty covered "a small service component [and] items."⁶⁴ As part of its determination, the OIG explicitly acknowledged that the proposed arrangement carried

⁶⁰ However, the OIG has considered some arrangements that do not involve federal health care programs to be problematic precisely because they exclude federal health care programs. For example, the discount safe harbor *excludes* from the definition of "discount" "a reduction in price applicable to one payer but not to Medicare, Medicaid, or other federal health care programs." See 42 C.F.R. § 1001.952(h)(3)(iii). In 1999, OIG clarified this exclusion as being necessary to "protect against abusive arrangements in which remuneration in the form of discounts on items or services for private pay patients is offered to a provider to induce referrals of Federal health care program patients." See 64 Fed. Reg. 63,518, 63,528 (Nov. 19, 1999).

⁶¹ It is also important to acknowledge the operational complexity for providers to separate care involving private and public payers in implementing new steps in health care delivery. As a result, many providers and payers may be unwilling to invest the resources to isolate commercial patient populations from Medicare/Medicaid populations. Payers may also prefer to have managed Medicaid and Medicare patients included in their commercial contracts with manufacturers. This type of separation also deters the type of holistic practice transformation that serves as the underpinning of more sophisticated and cost-saving VBP arrangements.

⁶² It is worth noting that state anti-kickback laws may reach strictly private VBP arrangements even when the federal AKS does not.

⁶³ Letter to Rep. Jim McDermott by Secretary Kathleen Sebelius, October 30, 2013, <http://www.hiregulation.com/files/2013/10/The-Honorable-Jim-McDermott.pdf>.

⁶⁴ OIG Advisory Opinion No. 01-8 (July 10, 2001).

potential to benefit patients and federal healthcare programs through its innovative design.⁶⁵ In a separate example, OIG similarly determined that a proposed arrangement not protected by the warranty safe harbor nevertheless contained sufficient “safeguards” to protect against the risk of fraud and abuse.^{66,67}

As these examples suggest, the “risk assessment” that OIG applies in the absence of safe harbor protection has been adapted to some extent to innovations in contracting and care, reflecting OIG’s understanding of the broader healthcare environment. With increasing pressures to find innovative ways to provide access to new treatments while also limiting overall costs, it is reasonable to expect that OIG’s risk assessments could be more receptive to contractual safeguards that reduce risk for program abuse and higher costs by promoting value-based care.^{68,69}

In short, the AKS’ obstacles to the development and adoption of VBP arrangements for innovative drugs and technologies in the context of Federal programs, like Medicare and Medicaid, were designed for a healthcare system that was susceptible to exploitation based on the volume of billable items and services. As healthcare reform increasingly shifts towards value-based reimbursement, however, the limitations of the AKS for VBP arrangements are becoming increasingly visible.

Yet, the AKS does not preclude all VBP arrangements. In addition to limiting contracts that may implicate AKS issues to their commercial patient population, manufacturers are also free to seek Advisory Opinions and appeal to the OIG’s risk assessment test by demonstrating adequate safeguards that protect the VBP arrangement against fraud and abuse. The problem for policymakers hoping to encourage VBP arrangements is that the Advisory Opinion process is a time consuming, limited, and relatively costly way to analyze risk from a company perspective. In the absence of further policy changes, the AKS will continue to serve as a very real barrier for the adoption and expansion of major VBP arrangements, particularly involving Medicaid and Medicare patients.

⁶⁵ *Id.*

⁶⁶ OIG Advisory Opinion No. 02-6 (May 22, 2002).

⁶⁷ More recently, the OIG found that a proposed arrangement involving replacement of a drug that became “spoiled” due to the customer’s inability to administer to the patient for certain reasons did not fall within the warranty safe harbor. However, the OIG declined to impose administrative sanctions because, among other things, the proposed arrangement “could increase patient safety and quality of care.” See OIG Advisory Opinion No. 17-03, at 5 (Aug. 25, 2017).

⁶⁸ For example, in Advisory Opinion 01-8, OIG commented on value and a proposed arrangement that did not satisfy an AKS safe harbor by stating: “In many respects, global payments are intended to encourage Facility operators to re-engineer the delivery of care to reduce costs and increase quality. *Given the absence of any identifiable opportunity for abuse, we are reluctant to chill innovative and potentially beneficial arrangements.*” (emphasis added).

⁶⁹ However, we are cautious of overstating OIG’s risk assessment flexibility since it is impossible to know precisely how the OIG may perceive the risks and benefits of a specific arrangement. Additionally, many manufacturers may find pursuing an Advisory Opinion unattractive considering the costs and the risk of exposure of sensitive business information.

Medicaid Best Price Requirement

The Medicaid Drug Rebate Program's Best Price calculation (MBP) requirement is another regulatory concern that stakeholders commonly cite as impeding the development and adoption of innovative VBP arrangements involving pharmaceutical products. Unlike other price reporting requirements, drug manufacturers are especially concerned about MBP implications of VBP arrangements because a single contract could affect payment across the entire Medicaid market.

Background on the MBP Requirement

In order for manufacturers to have outpatient drugs covered by the Medicaid program, they must participate in the Medicaid Drug Rebate Program (MDRP). The manufacturer does so by executing the Medicaid drug rebate agreement, pursuant to which the manufacturer agrees to pay a rebate to each state Medicaid program for its covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program. The rebate amount is based on pricing data reported by the manufacturer on a monthly and quarterly basis. These data include the AMP and, in the case of innovator products, the Medicaid Best Price (MBP). The rebate formula for innovator drugs consists of two components, the basic rebate and the additional rebate (also referred to as the inflation penalty). The basic rebate for innovator drugs is equal to the greater of (1) AMP times 23.1 percent and (2) AMP minus best price.⁷⁰

MBP is broadly defined to include “the lowest price” made available by the manufacturer to “any wholesaler, retailer, provider, health maintenance organization, nonprofit entity, or governmental entity within the United States,” although there are important exclusions discussed in more detail below.⁷¹ In its implementing regulations, CMS stated that best price would be determined “on a unit basis without regard to package size, special packaging, labeling, or identifiers on the dosage form or product or package.”⁷² It is worth noting that in defining “best price,” the statute is silent on whether best price must be measured on a “unit basis”.⁷³ CMS established the per-unit measurement as the standard for best price. Unlike AMP, MBP is not an average. It could be set by a single transaction in the quarter—namely the transaction that resulted in the lowest price “made available” by the manufacturer—unless an exception applies. If a drug's best price is lower than 23.1% below AMP, then the best price generates a higher basic rebate which applies to all Medicaid utilization of the drug in that quarter.

⁷⁰ The rebate percentage is 17.1 percent for certain clotting factors or products approved exclusively for pediatric indications. 42 U.S.C. § 1396r-8(c)(1)(B); 42 C.F.R. § 447.509(a)(1).

⁷¹ 42 U.S.C. § 1396r-8(c)(1)(C)(i).

⁷² 42 C.F.R. 447.505(d)(2).

⁷³ 42 U.S.C. § 1396r-8(b)(3)(A)(i)(II) establishes a requirement for manufacturers to report their “best price.” But in defining best price, the statute cross references subsection (c)(1)(C). Subsection (c)(1)(C) defines “best price” as the “lowest price available from the manufacturer during the rebate period...” Notably, it does not specify how “best price” is to be measured; it is silent on this matter.

How the MBP Requirement Impedes VBP Arrangements

The MBP requirement would appear to mean that if total discounts on any unit of the drug involved in a VBP arrangement result in a MBP that is lower than 23.1 percent below AMP, the manufacturer would be required to extend that discount to all of Medicaid. This would also have the ancillary effect of also lowering the so-called “Section 340B” ceiling price⁷⁴ for the drug. Because of these systemic impacts on a manufacturer’s Medicaid payments, MBP is widely viewed as limiting the discounts that can be provided in VBP arrangements⁷⁵. In particular, MBP could be triggered by an advanced outcome-based contract (OBC), in which the manufacturer would give back a large portion of payment when desired outcomes are not achieved.

To illustrate how MBP may hinder a VBP arrangement, consider an arrangement between a drug manufacturer and a payer wherein the manufacturer agrees to a tiered rebate structure that depends on the drug meeting a range of agreed-upon clinical outcomes. Different combinations of outcomes could yield different rebates, based on negotiations between the manufacturer and provider/payer. For example, a failure to meet outcomes X, Y, and Z would result in a rebate of 70 percent, while a failure to meet Y and Z would result in a rebate of 30 percent. But any rebate greater than 23.1 percent would require the manufacturer to offer the same rebate in all Medicaid contracts, even though the VBP arrangement was intended to be limited and offered in conjunction with new opportunities for value creation not present in FFS contracts.

Although other related price reporting obligations such as AMP and Average Sales Price (ASP) are not the focus of this White Paper, they too can similarly discourage the development and adoption of substantial VBP arrangements. This is particularly true for orphan drugs because these drugs will have fewer patients across which to spread their price, so that even a small number of VBP arrangements could lead to high volatility in Medicaid reimbursement. Even for more widely prescribed drugs, uncertainty about impacts on average price calculations may be a barrier to advanced VBP contracts with substantial links to outcomes.

Identifying Existing Flexibility within the MBP Requirement for VBP Arrangements

Although the MBP requirement is far-reaching, there are also broad exclusions from its purview.⁷⁶ Two of the most prominent exclusions involve the fact that the MBP, and the

⁷⁴ The 340B ceiling price refers to the maximum amount that a manufacturer can charge a covered entity for the purchase of a 340B covered outpatient drug. The 340B ceiling price is statutorily defined as the Average Manufacturer Price (AMP) reduced by the rebate percentage, which is commonly referred to as the Unit Rebate Amount (URA), available at <https://www.hrsa.gov/opa/updates/2015/may.html>.

⁷⁵ Tara O’Neil Hayes, “Current Impediments to Value-Based Pricing for Prescription Drugs,” American Action Forum (June 2017), available at <https://www.americanactionforum.org/wp-content/uploads/2017/06/2017-06-05-Rx-Value-based-pricing-and-policy-impediments-FINAL.pdf>; “Value-Based Pricing in Pharmaceuticals,” Global Strategy Group – KPMG (2016), available at <https://assets.kpmg.com/content/dam/kpmg/xx/pdf/2016/10/value-based-pricing-in-pharmaceuticals.pdf>.

⁷⁶ 42 C.F.R. 447.505(c)(1)(19).

obligation to report AMP and ASP, simply do not apply to medical devices because they are not “covered outpatient drugs.” Moreover, prices negotiated by prescription drug plans under Medicare Part D or by a Medicare Advantage Prescription Drug Plan under Medicare Part C are also excluded from the definition of “best price.”⁷⁷ This exclusion is substantial. In 2013, gross spending on outpatient prescription drugs under Part D amounted to \$103.7 billion, making it one of the largest markets for outpatient prescription drugs.⁷⁸ As a result, manufacturers may offer rebates in VBP contracts involving drugs dispensed to Part D enrollees without triggering the MBP requirement.⁷⁹

Furthermore, the MBP statute allows considerable flexibility to enter into VBP arrangements within the Medicaid program itself. In particular, the “best price” definition excludes prices offered to any given state that include “supplemental rebates,” as authorized by CMS.⁸⁰ This means that manufacturers can enter into innovative VBP arrangements with specific states without undermining their best price. Indeed, CMS has expressly encouraged states to enter into these types of arrangements, and has specifically proposed that Medicaid state agencies could align their fee-for-service preferred drug lists with the state’s Medicaid managed care organizations (MCOs) formularies for certain classes of drugs in order to collect supplemental rebates on those drugs and avoid changing their (the state’s) approved state plan.⁸¹ Regardless of the specific methodology that states employ to take advantage of the supplemental rebate exception to MBP, the exception does present manufacturers with some flexibility to develop and test VBP arrangements in partnership with state Medicaid agencies without implicating MBP.

Although the “best price” exclusions may enable some flexibility under the Medicare and Medicaid programs, MBP arguably poses the largest barrier in the commercial market. However, as other commentators have noted, the MBP requirement is manageable to some extent even in commercial arrangements.⁸² For example, in some circumstances, stakeholders could establish indication-specific pricing for chemically-similar drugs with different National Drug Codes (NDCs) that would set rebates *within* the 23.1 percent threshold for triggering MBP.⁸³ This threshold provides some leeway to tie the price of the drug to its indication-specific efficacy or to outcomes.⁸⁴ However, more advanced VBP models that tie a large share of reimbursement to outcomes are less likely to fall within the threshold.

⁷⁷ 42 C.F.R. 447.505(c)(6).

⁷⁸ MEDPAC June 2016 Report, Chapter 10 – Prescription Drugs, at 166.

⁷⁹ Admittedly, though, this flexibility applies to negotiations between manufacturers and *plans*, not providers.

⁸⁰ 42 C.F.R. 447.505(c)(7).

⁸¹ See Medicaid Drug Rebate Program Notice – For State Technical Contacts, CMS No. 176 (July 14, 2016).

⁸² Sachs, Rachel, Bagley, Nicholas and Lakdawalla, Darius N., “Innovative Contracting for Pharmaceuticals and Medicaid’s Best-Price Rule” (April 28, 2017). Journal of Health Politics, Policy and Law, Forthcoming; U of Michigan Public Law Research Paper No. 549. Available at SSRN: <https://ssrn.com/abstract=2959939> or <http://dx.doi.org/10.2139/ssrn.2959939>.

⁸³ See *id.* at 4 (using the examples of sildenafil, which received separate FDA-approvals and unique NDCs for Viagra and Revatio). However, this is not a common practice.

⁸⁴ See *id.*

Commentators have also proposed workarounds within the current MBP environment for OBCs. Rebates under OBCs are not tied to any particular indication, but rather to whether specific patients in a covered population achieve certain clinical outcomes. One possible way to align an OBC with MBP requirements is to calculate relevant performance measures across a patient population, as opposed to calculating them on a per-patient basis.⁸⁵ For example, instead of offering a 50 percent rebate on one drug prescription because a particular patient fails to meet the specified clinical outcomes, the manufacturer could offer a rebate according to how many aggregate patients on the drug in the covered population achieve the specified clinical outcomes.⁸⁶ Although this workaround would trigger MBP if the “population-based outcome” rebate exceeds 23.1 percent across the affected population, this approach avoids triggering MBP because of a *single* patient’s failure to achieve expected clinical outcomes. In those cases where a single contract does not cover enough patients to mitigate the potential for sharp swings in MBP (for example, a contract with a specific plan or provider for an orphan disease might cover only a small number of patients), manufacturers could argue to CMS that a network of contracts with similar or identical discount provisions represents the relevant basis for calculating price.⁸⁷

The MBP definition also excludes “free goods” so long as the free goods are not contingent upon any purchase requirement.⁸⁸ The manufacturer could structure the transaction such that a “sale” does not occur until, for example, the patient responds to the therapy. If the patient fails to respond to the therapy, the manufacturer would not demand payment—the buyer would receive the therapy for free. Arguably, the drug provided under those circumstances would be a “free good” not contingent on any purchase requirement, and therefore arguably the transaction would not be relevant for the BP calculation. The specific terms of the arrangement would have to be carefully devised to support and strengthen such an argument.

⁸⁵ *Id.* at 6.

⁸⁶ The bundled sales provision at 42 C.F.R. § 447.502 may offer such flexibility by allowing manufacturers to modify how they report pricing, including best price. Under the regulations, a “bundled sale” includes discounts or other price concessions that are conditioned upon a “performance requirement.” *See id.* The discounts in a bundled sale must be “unbundled” through the proportional allocation of discounts across the bundled arrangement. This potentially allows for a manufacturer to offer large rebates for Drug X when patients fail to meet a specified outcome (i.e. the performance requirement), but because these larger rebates would be averaged with the smaller rebates representing instances where patients *did* meet clinical outcomes, the *reported* price is higher than it would otherwise be, thereby possibly avoiding triggering MBP. However, there is significant uncertainty in interpreting the bundled sales provision this way because CMS’ interpretation of the term “performance requirement” has not included performance of the *product*, such as its success in achieving clinical outcomes. Instead, CMS has used the term to describe the performance obligations of *contracting parties*, such as where the manufacturer conditions a rebate for a drug on the “performance requirement” of the payer placing the drug on its formulary. *See* “Medicaid Program; Prescription Drugs,” 72 Fed. Reg. 39142, 39158 (July 17, 2007).

⁸⁷ Of course, these innovative contractual arrangements assume that the operational framework for their successful implementation exists, which is not always the case.

⁸⁸ 42 C.F.R. § 447.505(c)(13).

These creative interpretations of MBP requirements in the setting of VBP contracts can help advance the use of VBP contracts where payments depend substantially on outcomes. However, in the absence of more clarity from CMS about the appropriateness of these interpretations, manufacturers will likely continue to perceive risk that the government would view such transactions differently, particularly limiting the use of more advanced VBP contracts. The legal uncertainty surrounding the implications of MBP remains a significant barrier to the development and adoption of VBP arrangements.

Regulatory and Legislative Solutions

The limited flexibilities identified above may be enough to enable some stakeholders to pursue meaningful VBP arrangements. But we expect that most stakeholders are more risk-averse and will avoid widespread and advanced investments in VBP arrangements that move substantially away from volume-based contracts until they have more clarity about the regulatory implications of these new payment models, particularly related to the three key regulatory issues we have addressed in this White Paper. Below, we offer specific regulatory and legislative proposals for each of the three issues that could enable a more substantial shift from FFS to value-based payment. In all cases, we emphasize the importance of considering the spectrum of VBP arrangements in applying new regulatory approaches. While relatively limited VBP contracts appear to be feasible without significant regulatory and legislative changes, widespread shifts to advanced VBP arrangements are likely to require a regulatory structure that is better aligned, with payment arrangements based mainly on outcomes and value rather than volume.

Regulatory Proposals

FDA Regulation of Off-Label Communication

FDA's proposed policy on HCEI dissemination greatly expands a manufacturer's ability to share HCEI about an approved drug with payers without increasing their regulatory risk for violating FDA regulations. The draft guidance permits some proactive, pre-approval communications about an investigational drug or device, but does not address a manufacturer's ability to share HCEI about unapproved uses of already marketed products, or many important areas of information exchange with providers in VBP arrangements. The following additional proposals for FDA would address these issues and enhance the adoption of VBP arrangements as well as the development and use of real-world evidence needed for such arrangements.

Prioritize Finalizing the HCEI Draft Guidance and Provide Additional Clarification. Questions remain with respect to permissible manufacturer communication of HCEI as to parties engaged in VBP arrangements. The draft guidance leaves ambiguity about whether the restriction on sharing HCEI with "health care providers in charge of prescribing" would apply to emerging VBP arrangements between manufacturers and healthcare providers. Failure to include healthcare providers that engage in risk-bearing activities could hinder value-based arrangements that align manufacturers and providers. Such aligned alternative payment models may be increasingly important for new models of care delivery involving medical products to succeed.

Including providers in significant VBP arrangements aligns with Congressional intent in the Cures Act's extensive modification of the definition of HCEI, where information may be shared with "a payor," . . . or other similar entity with *knowledge and expertise in the area of health care economic analysis*, carrying out its *responsibilities for the selection of drugs for coverage or reimbursement*." Like payers and public health decision makers, health-care providers that are substantially at risk in emerging alternative payment models should have access to HCEI.

Implement a Safe Harbor for VBP Arrangements. RWE and off-label information outside the context of HCEI will continue to be an issue as VBP arrangement contract participants seek to address care issues beyond FDA labeled indications to improve quality, outcome, and cost metrics. FDA should provide a safe harbor for VBP arrangements that utilize outcome and other clinical value-defining metrics that may fall outside the scope of qualifying HCEI dissemination under the payer guidance. Additionally, FDA's current draft payer guidance has proposed that HCEI dissemination meet promotional requirements under 21 CFR 314.81(b)(3)(i), such as pre-dissemination submission using Form FDA 2253. In light of the sensitive nature of commercial negotiations, a safe-harbor should exclude commercial negotiations from promotional reporting requirements.

Permit Dissemination of HCEI Related to an Investigational Intended Use. FDA should permit parties engaged in a VBP arrangement to share HCEI about an unapproved indication for an approved drug that is undergoing clinical investigation. This suggestion aligns with FDA's proposal to create a new safe harbor to permit sharing certain information about a new investigational drug or device in order to assist payers and public health decision makers with budget planning and formulary design. The negotiation process for VBP arrangements is often time-consuming and involves many operational challenges. As with the safe harbor for new drugs and devices, if sponsors are restricted from starting the negotiation process prior to a product's approval for a new indication, there is a risk that patients will be restricted in gaining access to emerging innovative therapies coverage because VBP arrangements that tie payments to results cannot readily be implemented. This is especially true for breakthrough therapies that are approved under expedited pathways.

Leverage 21st Century Cures Authorities to Facilitate the Development of VBP Arrangements with RWE. In light of the importance of RWE to VBP arrangements and current operational challenges associated with defining value, FDA should leverage the newly acquired Cures authorities to facilitate VBP arrangements development. Under new section 505(f) entitled "utilizing real world evidence," FDA must develop a program evaluating the potential use of RWE to support approvals of new indications for previously approved drugs under section 505(c) and post approval study requirements. As part of this initiative, FDA must issue draft guidance by December 13, 2018 establishing a new framework for section 505(f), which includes information on sources of RWE, gaps in data collection activities, methodologies for collection and analysis, RWE priorities, RWE remaining challenges, and "potential pilot opportunities that the program established." A potential pilot program involving VBP arrangements that substantially depend on outcomes can facilitate FDA's understanding of the changing landscape in health care and the data and operational challenges currently involved

with identifying, collecting, and analyzing real world data. This type of pilot may also inform FDA about the use of enforcement discretion or the creation of a safe-harbor for VBP agreements that generate meaningful evidence. Additionally, any RWE-based pilots should generally be used as an opportunity to not only inform regulatory decision making about labeling, but also to help inform FDA's thinking on the use of RWE in manufacturer communications with payers and providers.

Promulgate Regulations on Off-label Promotion. FDA should promulgate regulations with respect to its policies on off-label promotion and permissible manufacturer communications, at least in the context of VBP arrangements that represent a substantial shift away from volume-based payment. Failure to do so leaves parties with interests in VBP arrangements with less clear support from a non-binding guidance document and a piecemeal regulatory landscape to determine what information would be permissible or impermissible to communicate.

The Anti-Kickback Statute

The AKS' limitations on VBP arrangements could be addressed most clearly through OIG's creation of a distinct safe harbor for VBP arrangements that represent a substantial shift from the volume-based payment incentives motivating much of current AKS requirements. Short of that solution, however, the OIG could take a range of steps described below to facilitate VBP arrangements. Although many of the AKS proposals discussed below are interrelated, some would require more significant work to implement, and they can be pursued independent of each other.

Reinterpret "Volume or Value of Any Referrals" in the Context of VBP Arrangements.

Currently, many safe harbors do not protect an arrangement that takes into account the volume or value of any referrals. For example, the provision of EHR software cannot directly take into account the volume or value of business generated between the contracting parties.⁸⁹ This broad prohibition could stifle innovative VBP arrangements that include, for example, incentive payments for improving quality, which may be partially or entirely tied to "volume" or "value of referrals" involving providers and medical products in coordinated-care arrangements intended to enable success in improving outcomes and lowering total costs of care. CMS/OIG should issue sub-regulatory guidance formalizing the OIG's risk-assessment for certain safe harbors in order to allow flexibility to enter into VBP arrangements, where the benefits of the arrangements to coordinate and improve care outweigh potential risks. This may be more likely in cases where the VBP arrangement is heavily results-based, in which payments depend significantly on limiting total costs of care and improving outcomes. Such outcome impacts could provide strong evidence that the arrangement will not lead to unnecessary program spending.

Reinterpret "Fair Market Value" in the Context of VBP Arrangements. Currently, CMS/OIG impose a fair market value requirement on most safe harbors. This requires contract terms to

⁸⁹ 42 C.F.R. § 1001.952(y)(5).

reflect an “arms-length” transaction. However, VBP arrangements may or may not always reflect fair market value, such as where a manufacturer/payer agrees to provide reduced-priced data analytic services in conjunction with its product, because such analytics can improve outcomes in the context of a VBP arrangement. Therefore, as described above, CMS/OIG should issue sub-regulatory guidance clarifying that it will modulate the fair market value requirement when evaluating VBP arrangements. Alternatively, CMS could revisit specific safe harbors through rulemaking and clarify that the requirement will be modulated in the context of VBP arrangements.

Revise Existing Safe Harbors to Facilitate VBP Arrangements. As discussed above, there are critical components of VBP arrangements that fall outside the protection of existing safe harbors because of how narrowly the safe harbors have been designed. To encourage further development and adoption of VBP arrangements, the OIG could pursue targeted reforms that change specific limiting provisions of select safe harbors. These revisions would be subject to notice-and-comment rulemaking to ensure robust stakeholder input. As a starting point, the revisions could include:

- Revise the Electronic Health Records Items and Services safe harbor⁹⁰ to allow for a greater range of technologies (information sharing, data analytics, cybersecurity assistance, etc.) and related training in the context of VBP arrangements. In addition, the revision would allow the donation of these technologies to be contingent on purchase of given product, and potentially include ability to restrict use depending on the type of technology. It would also extend this safe harbor permanently (it ends in 2021) for practices not currently using an electronic health records system.
- Revise the Warranties safe harbor⁹¹ to allow a manufacturer to reimburse providers/payers with ancillary costs that are associated with a product’s proximate failure to perform as expected. Such manufacturer remuneration could be provided in-kind or otherwise in connection with products that fail to achieve a pre-determined set of clinical or economic outcomes in a VBP contract.
- Revise the Personal Services/Management safe harbor⁹² to allow more flexibility in entering into these contracts, particularly by removing the requirement that these contracts not be for less than one year. Additionally, the revision would acknowledge that personal services/management associated with VBP arrangements are not business referrals.
- Revise the Discounts safe harbor⁹³ to allow differential pricing associated with the selling of one product or service at reduced charge to induce the purchase of a different product or service. As discussed above, the safe harbor permits this only if the same program using the same methodology reimburses the goods and services. This requirement is unduly restrictive in the context of VBP arrangements. Additionally, the time horizon for when a

⁹⁰ 42 C.F.R. § 1001.952(y).

⁹¹ 42 C.F.R. § 1001.952(g).

⁹² 42 C.F.R. § 1001.952(d).

⁹³ 42 C.F.R. § 1001.952(h).

buyer must include a discount in their cost report should be extended to several years in order to encourage VBP contracts to support better evidence and greater long-term benefits of therapies. Finally, OIG should clarify that the safe harbor extends to commercial payers as well.

Establish a VBP Arrangement Safe Harbor. The targeted changes to existing safe harbor provisions discussed above would facilitate VBP arrangements, but do not amount to a more comprehensive approach designed specifically to advance VBP arrangements that substantially differ from traditional FFS payments, from a program integrity standpoint. In particular, the OIG could aim to develop a distinct, comprehensive safe harbor that is designed to address the most common elements underlying desirable VBP arrangements. The OIG should subject the proposal to notice-and-comment rulemaking and incorporate robust stakeholder input into the safe harbor's design. As a starting point, the safe harbor would:

- Recognize coordination and integration between stakeholders that specifies clear, meaningful, and substantial goals for improving patient outcomes and reducing costs, including a significant share of payments tied to measurable performance benchmarks/outcomes.
- Recognize that the bundling of items and services may be integral to the operational and/or financial success of a VBP arrangement.
- Require up-front agreement on the terms that comprise the VBP arrangement and to the extent some terms are contingent upon the occurrence or non-occurrence of a future event, those terms should be adequately identified in writing.
- Recognize that time-horizons in a VBP arrangement can be long-term, and that arrangements that encourage improving long-term impacts may be particularly challenging to implement under current rules.
- Recognize that VBP arrangements may include gainsharing and risk-sharing as the primary financing mechanism, including risk-sharing around total costs of care.
- Permit flexibility in selecting pre-specified clinical endpoints consistent with FDA rules and guidance.

Medicaid Best Price

MBP requirements provide some important flexibilities for VBP reforms.⁹⁴ However, additional reforms to the MBP could enhance legal certainty for stakeholders and spur innovation in more advanced VBP arrangements.

Reinterpret The Bundled Sales Provision. As discussed above,⁹⁵ the bundled sales provision that enables manufacturers to allocate discounts proportionately across a qualified bundled sale could serve as a regulatory foothold for the agency to encourage VBP arrangements. In particular, CMS should issue clarifying guidance that a “performance requirement” under the bundled sales provision could include performance of the product itself, as opposed to the

⁹⁴ See Sachs et al., *supra* note 82.

⁹⁵ See *infra* n. 86.

performance obligations of the contracting parties. CMS could do this through a manufacturer release by positing a bundled sales example involving a VBP arrangements wherein the manufacturer agrees to offer the base rebate if a given drug is placed on formulary, and an additional outcome-based rebate if the drug fails to meet specified outcomes. Through such a manufacturer release, CMS could quickly enable manufacturers, as part of VBP arrangements, to allocate outcome-based discounts across patient populations, thereby avoiding the triggering of MBP through a single sale.

Clarify that Rebates Based on Value Negotiated by Medicaid Managed Care Organizations Do Not Trigger MBP. In its July 2016 guidance to states, CMS was not clear whether Medicaid MCOs could negotiate rebates in VBP arrangements with manufacturers without concern of implicating MBP.⁹⁶ CMS should issue sub-regulatory guidance clarifying that Medicaid MCOs can negotiate their own rebates related to outcomes and other measures of value while falling within the supplemental rebate exception. CMS could require state Medicaid agencies to review and approve Medicaid MCO-negotiated rebates. If CMS determines that its regulations require states to negotiate rebates on behalf of MCOs in order to satisfy the requirements of the supplemental rebate exception to the MBP, it may be appropriate to pursue notice-and-comment rulemaking to change the regulatory text itself.⁹⁷ If the agency determines it lacks statutory authority to authorize Medicaid MCOs to negotiate their own rebates under the supplemental rebate exception, Congress should step in and legislatively authorize these arrangements.⁹⁸ Allowing Medicaid MCOs to negotiate VBP arrangements independently of the state Medicaid agency would inject more flexibility into the types of VBP arrangements that drug manufacturers and Medicaid MCOs could enter into for Medicaid managed care populations, especially as managed care increasingly penetrates state Medicaid programs.

Modify Basis of Measurement For MBP In The Context of VBP Arrangements. One regulatory reform, which is also proposed by Sachs et al., that CMS could adopt in order to encourage VBP arrangements involves revising the regulatory requirement that best price be calculated “on a unit basis.”⁹⁹ CMS may be able to establish an alternative method to reporting best price that does not rely on a per-unit basis.¹⁰⁰ A per-unit basis approach does not align well with VBP reforms that do not pay primarily based on volume. For example, a population-based

⁹⁶ See Medicaid Drug Rebate Program Notice – For State Technical Contacts, CMS No. 176 (July 14, 2016) (“[W]e urge that states consider negotiating supplemental rebates with manufacturers for some or all of their Medicaid managed care drug claims. Before negotiating supplemental rebates on managed care drug claims, states should determine the impact of their decision to collect supplemental rebates on their contracts with managed care organizations”).

⁹⁷ 42 C.F.R. § 447.505(c)(7) (“Rebates under the national rebate agreement or a CMS-authorized supplemental rebate agreement paid to State Medicaid Agencies under section 1927 of the Act.”).

⁹⁸ CMS established the supplemental rebate exception as part of its interpretation of § 1927(c)(1)(C)(ii)(I) of the SSA, which requires rebates paid by manufacturers under section 1927 to be excluded from the calculation of best price. See “Medicaid Program; Prescription Drugs,” 72 Fed. Reg. 39142 (July 17, 2007).

⁹⁹ See Sachs et al., *supra* note 82, at 5-6.

¹⁰⁰ There are questions regarding CMS’ statutory authority to pursue this approach, however. As a result, legislative action may be required.

alternative payment model may base reimbursement for a drug on a per-member (PMPM) or per-patient per-month (PPPM) amount, as opposed to the number of units actually sold. Under the current system, such an arrangement is discouraged because manufacturers are required to reduce a PMPM/PPPM arrangement to its “per-unit” basis for reporting purposes. This may produce a rebate greater than 23.1 percent of AMP and thereby trigger MBP, especially among smaller patient populations. As a result, the per-unit basis requirement effectively discourages stakeholders from pursuing innovative VBP arrangements that depart from setting price on the volume of goods sold.

Establish Section 402 Demonstrations for VBP Arrangements. CMS should exercise its waiver authority under section 402 the Social Security Amendments of 1967¹⁰¹ to establish a demonstration that would enable VBP arrangements. Section 402 authorizes HHS/CMS to waive any provision in Title XIX (Medicaid) “insofar such requirements relate to...reimbursement or payment only for such items or services as may be specified in the experiment.”¹⁰² Historically, CMS has not relied on section 402 to conduct Medicaid-only demonstrations and the agency has opted instead to use section 402 to establish Medicare/Medicaid dual-eligible demonstrations in conjunction with Medicaid section 1115 waivers.¹⁰³ However, the language of section 402 contemplates a demonstration/experiment that could involve a waiver of the MBP requirement (and other price reporting obligations) for the purposes of facilitating VBP arrangements.¹⁰⁴

Legislative Solutions

FDA Regulation of Manufacturer Communications

While the regulatory approaches described above could diminish the need for legislative reforms related to FDA regulation of manufacturer communications and implications on VBP arrangements, policy makers could also consider legislative solutions to encourage the broader adoption of value-based arrangements in the U.S.

Establish a Safe-Harbor for Pre-Approval Communication of HCEI. The Pharmaceutical Information Exchange (PIE) Act of 2017 (H.R. 2026) is one example of legislation that would create a legislative safe harbor for pre-approval communications involving HCEI or “scientific

¹⁰¹ Codified at 42 U.S.C. 1395b-1.

¹⁰² 42 U.S.C. § 1395b-1(b).

¹⁰³ 42 U.S.C. § 1315; *see also* Robert Schmitz et al., “Evaluation of Medicare Advantage Special Needs Plans: Summary Report,” Mathematic Policy Research, Inc., (Sept. 30, 2008), *available* at <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Reports/downloads/Schmitz2008.pdf> (discussing the transition from dual-eligible demonstrations to Medicare Advantage special needs plans for Minnesota Senior Health Options demonstration, Minnesota Disability Health Options demonstration, and Massachusetts Senior Care Options demonstration).

¹⁰⁴ There are three different provisions in sec. 402 that may serve as the predicate for such a demonstration. *See* 42 U.S.C. 1395b-1(a)(1)(A), (C), and (F). Sec. 402 waiver authority is potentially much broader than section 1115 waiver authority, which does not include section 1927 of the Social Security Act wherein the MBP requirement is housed.

information.”¹⁰⁵ It would apply the same HCEI safe-harbor standards established under the Cures Act to HCEI and scientific information with respect to an investigational drug or a new investigational use (i.e., following the “CARSE” standards). Similar to the regulatory proposals described above, an approved product with a new unapproved indication would only qualify for the safe harbor if a supplemental application has already been filed, which mitigates concerns that permitting this type of communication would disincentivize premarket review and submissions. Indeed, encouraging the inclusion of requirements to develop evidence on additional indications as part of VBP arrangements could help advance FDA priorities related to improving real-world evidence related to currently off-label indications and claims. As stated previously, the ability to communicate HCEI preapproval with respect to both an investigational product and an investigational new use is exceedingly important for payers for budget planning and formulary design. With respect to VBP arrangements, the ability to communicate this information is even more vital in light of the operational challenges and time constraints involved with their implementation and execution.

Create Regulatory Certainty for Off-Label Information to Support Value-based Care Models.

Legislation could also include a safe harbor for parties engaged in VBP arrangements generally, such as for healthcare provider-manufacturer innovative VBP arrangements, particularly when those efforts will create more evidence related to claims that are currently off-label. Modifying the scope of the HCEI provision or adding a new provision under section 502(a) of the Federal Food, Drug and Cosmetic Act would resolve any ambiguity that currently exists under FDA’s currently proposed policy on HCEI communication. By creating a statutory carve out for only parties engaged in substantial VBP arrangements and alternative payment models, and by highlighting ways in which such arrangements could support the development of needed evidence, FDA may maintain its public health objectives of limiting dissemination of off-label information to prescribing providers and also create new incentives for the development and use of real-world evidence.

While regulatory solutions are available, FDA’s regulation of manufacturer communications about off-label information is an unsettled area. Recent case law on Constitutional limitations of FDA policy further compounds interpreting this framework. A legislative safe harbor involving VBP arrangement parties would create the most certainty for moving VBP reforms forward.

Anti-Kickback Statute

Establish Clear Policy Direction With Respect to VBP Arrangements. As discussed above, Congress has already provided the OIG with broad authority to establish safe harbors that could accommodate VBP arrangements. Congress could establish *statutory* safe harbors as it has done in the past, but this route seems inefficient considering that the OIG could administratively achieve the same result under its existing authority. Instead, Congress could

¹⁰⁵ “Scientific information” would broadly include “clinical and pre-clinical data and results relating to an unapproved drug therapy, or drug indication, or other condition of use being investigated or developed.”

make the AKS more amenable to VBP arrangements is through directing the OIG to issue or revise existing safe harbors in accordance with broad policy objectives to support VBP arrangements.

Medicaid Best Price

Establish Safe Harbors for MBP. Legislative action could increase the development and adoption of VBP arrangements. In particular, Congress could authorize CMS to create a specific process for exempting advanced VBP arrangements from the MBP requirement.

Modify Center for Medicare and Medicaid Innovation (CMMI) Statutory Authority. As a more limited step to support further development of these VBP exemptions, Congress could also expand Center for Medicare and Medicaid Innovation (CMMI) authority to waive section 1927 of the Social Security Act.¹⁰⁶ This would enable CMMI to create VBP arrangement demonstrations that protect manufacturers from the MBP and other related requirements. In conjunction with this expanded waiver authority, Congress could authorize CMMI to test demonstrations that are designed to spur private sector innovation,¹⁰⁷ as others have suggested.¹⁰⁸ For example, CMMI could propose a limited demonstration that tests a VBP arrangement involving primarily or exclusively non-Medicare and non-Medicaid beneficiaries. These demonstrations would waive the MBP and other related requirements, although it is important to note that absent Congressional authorization to make successful waivers permanent, the MBP and related requirements would reactivate after the CMMI demonstration concludes.

Modify Basis of Measurement for MBP through Legislation. While a regulatory proposal was presented above on this topic, CMS' regulatory efforts to modify the per-unit basis for reporting MBP could face significant statutory limitations. As a result, Congress may need to authorize alternative methodologies for implementing the MBP requirement. In particular, Congress could statutorily establish an alternative methodology for calculating MBP that is more sensitive to VBP arrangements, such as requiring that MBP be calculated on an aggregate basis across a patient population. This would resonate with the shift towards personalized medicine by allowing for sophisticated VBP arrangements that focus on aligning financial incentives with using a specific therapy in a way that maximizes patient responses in a population, not with maximizing use of the therapy.

Conclusion

The coming decade promises an unprecedented wave of novel technologies and transformative therapies that have the potential to dramatically improve the lives of patients. Ensuring access

¹⁰⁶ CMMI's waiver authority extends to all of Medicare and select provisions of Medicaid, none of which include § 1927. See 42 U.S.C. § 1315a(d)(1).

¹⁰⁷ Currently, CMMI is authorized to only test demonstrations that address deficiencies or potentially avoidable expenditures in the Medicare or Medicaid program. See U.S.C. § 1315a(b)(2)(A).

¹⁰⁸ See Sachs et al., *supra* note 82, at 8-9.

for patients while reducing avoidable healthcare costs will be critical to realizing the full value of these technologies and therapies. Given the struggles of existing regulatory frameworks designed for fee-for-service payment to accommodate high-value use of these therapies, VBP arrangements are gaining traction as a means of better aligning the payment for technologies and therapies with the value they produce and to promote adequate patient access while avoiding unnecessary costs. Although VBP arrangements also continue to face operational and logistical challenges, we have described how legal and regulatory barriers impede their development and expansion, especially of VBP models that depart substantially from fee-for-service payment.

This White Paper has identified limited flexibility within three statutory and regulatory frameworks that stakeholders frequently cite as impediments to VBP: the FDA's regulation of manufacturer off-label communications, the AKS, and the MBP requirement. In light of the sparse flexibility and high compliance risk associated with pursuing VBP arrangements under current legal requirements, we recommend FDA, OIG, CMS, and Congress take certain steps to significantly foster legal certainty and incentivize the further development and adoption of VBP arrangements. Until these barriers are resolved, either through regulatory or legislative action, VBP arrangements may move forward slowly, within the constraints of these systems based primarily on volume-based payments, effectively hindering the high-value technological innovation and delivery system reform that more advanced VBP arrangements could achieve. Regulatory innovation to address these obstacles should match and support the innovations in 21st century technologies and healthcare organizations, by providing a clear pathway for aligning manufacturers and healthcare providers behind payment approaches that deliver better outcomes and avoid unnecessary healthcare costs.